Cystic Fibrosis a fight we must win

Press Release

04 August 2016

NHS FUNDING DECISION FOR IVACAFTOR (KALYDECO) FOR TWO TO FIVE YEAR OLDS

The implications of this week's judicial review around NHS funding for treatments are concerning for cystic fibrosis as it has created uncertainty around when children (two to five year olds with the G551D and eight rarer gating mutations*) will receive access to Ivacaftor (Kalydeco), a life-changing treatment for the condition. Ivacaftor is proven to significantly improve lung function and slow the progression of cystic fibrosis, reducing time spent in bed on intravenous antibiotics from five weeks to less than five days a year.

The first precision medicine in cystic fibrosis, Kalydeco, targets a mutation that only a little more than 4% of people with cystic fibrosis in the UK have. The drug has been shown to dramatically improve lung function and reduce hospital admissions, leading to hopes it could normalise life expectancy for those who could benefit from it.

This development does not suggest any risk to treatments that are already funded and prescribed by the NHS in England, but we are aware that it may cause a delay in the provision of Kalydeco for two to five year olds, and we will be working with the manufacturer and NHS England to find a solution. We will continue to do all we can to ensure access to this treatment as quickly as possible, particularly as NHS England have already accepted it as cost-effective.

Please be assured that we are doing everything we can to make sure that children receive this treatment as soon as possible.

Ends.

*Including those who have one of nine gating mutations in the cystic fibrosis transmembrane conductance regulator (CFTR) gene G551D, G1244E, G1349D, G178R, G551S, S1251N, S1255P, S549N and S549R.

Notes to Editors

- Cystic fibrosis is a life shortening genetic condition – only half will live to celebrate their fortieth birthday.
- The Cystic Fibrosis Trust is the only UK-wide charity dedicated to fighting for a life unlimited for everyone affected by cystic fibrosis.

- Two million people in the UK are carrying the faulty gene without realising it. You could be one of them. If two carriers have children, there's a one in four chance their child will have the condition, which slowly destroys the lungs and digestive system.
- Cystic fibrosis is an inherited disease caused by a faulty gene. This gene controls the movement of salt and water in and out of your cells, so the lungs and digestive system become clogged with mucus, making it hard to breathe and digest food.
- People with cystic fibrosis often look perfectly healthy. But it's a lifelong challenge involving a vast daily intake of drugs, time-consuming physiotherapy and isolation from others with the condition. It places a huge burden on friends and family, and the condition can critically escalate at any moment.
- To find out more about cystic fibrosis and the work of the Trust, visit our website www.cysticfibrosis.org.uk.
- To support our fight for a life unlimited by cystic fibrosis text BEATCF to 70500 to give £5 to the Cystic Fibrosis Trust. Terms.

Contact

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